
Pluripotent stem cells in neurodegenerative and neurodevelopmental diseases.

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Public Summary:

Scientific Abstract:

Most of our current knowledge about cellular phenotypes in neurodevelopmental and neurodegenerative diseases in humans was gathered from studies in postmortem brain tissues. These samples often represent the end-stage of the disease and therefore are not always a fair representation of how the disease developed. Moreover, under these circumstances, the pathology observed could be a secondary effect rather than the authentic disease cellular phenotype. Likewise, the rodent models available do not always recapitulate the pathology from human diseases. In this review, we will examine recent literature on the use of induced pluripotent stem cells to model neurodegenerative and neurodevelopmental diseases. We highlight the characteristics of diseases like spinal muscular atrophy and familial dysautonomia that allowed partial modeling of the disease phenotype. We review human stem cell literature on common neurodegenerative late-onset diseases such as Parkinson's disease and amyotrophic lateral sclerosis where patients' cells have been successfully reprogrammed but a disease phenotype has not yet been described. So far, the technique is of great interest for early onset monogenetic neurodevelopmental diseases. We speculate about potential further experimental requirements and settings for reprogrammed neurons for in vitro disease modeling and drug discovery.

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